An Observational, Non-Interventional, Multicenter, Multinational Registry of Patients With Atypical Hemolytic Uremic Syndrome: Initial Patient Characteristics

Christoph Licht,[1] Gianluigi Ardissino,[2] Gema Ariceta,[3] Jon Beauchamp,[4] David Cohen,[5] Larry A. Greenbaum,[6] Sally Johnson,[7] Masayo Ogawa,[8] Franz Schaefer,[9] Johan Vande Walle,[10] Véronique Fremeaux-Bacchi[11]

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INTRODUCTION

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- Plasma exchange and infusion (PE/PI) has been the treatment of choice for aHUS[1]; however, evidence suggests that PE/PI offers no significant benefit over simple supportive therapy.[2,3]
- Eculizumab (Soliris®; Alexion Pharmaceuticals, Inc., Cheshire, CT, USA), a terminal complement inhibitor, is a humanized monoclonal antibody that binds with high affinity to the human C5 complement protein, blocking the generation of pro-inflammatory C5a and C5b-9.[4]
- Eculizumab is the first approved treatment for aHUS in pediatric and adult patients.[4-7]
- The global aHUS patient registry (US National Institutes of Health ClinicalTrials.gov Identifier: NCT01522183) was initiated in April 2012 to prospectively capture postmarketing effectiveness and safety data on patients treated with eculizumab; the registry will record information on the progression of disease in all aHUS patients (whether treated with eculizumab or with other disease management strategies).
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  - The industry sponsor fosters relationships with academic partners, building credibility and scientific integrity, while also providing transparency and clear guidelines for publication.
- A single, global aHUS patient registry can maximize both physician and patient participation to best capture information on disease, safety, and efficacy data in a population with a very rare disease.

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- To report patient characteristics and describe important milestones achieved by patients enrolled in the aHUS registry from its inception (April 2012) through September 2013.

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- Collection and evaluation of safety and efficacy data specific to the use of eculizumab in patients with aHUS
- Time to first and subsequent occurrence of specified events
- Assessment of the long-term manifestations of TMA complications of aHUS; other clinical outcomes, including morbidity and mortality in patients with aHUS receiving eculizumab treatment or treated with other disease-management approaches

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- Data are collected at study enrollment and every 6 months thereafter and include the following:
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  - TMA complications
  - Associated treatments and concomitant medications
  - Clinical and patient-reported outcomes
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  - To follow each patient and assess long-term outcomes for a minimum of 5 years, information from patient medical records is entered via a secure web portal and maintained anonymously.

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  - Advise, counsel, and guide individuals on publications that utilize aHUS registry data and resources and/or use the aHUS registry name.
  - Review publication drafts before submission to journals or public release.

Inclusion for the Current Analysis

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  - Registry enrollment date, date of birth, and sex
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RESULTS

Patient Characteristics in Global aHUS Patient Registry

- **Tables 1–4** provide information on demographics, aHUS diagnosis, baseline clinical characteristics, and eculizumab treatment characteristics.

Countries Enrolling Patients into aHUS Patient Registry (as of September 25, 2013)

- Australia, 9.0% (n=19)
- Austria, 3.8% (n=8)
- Denmark, 0.5% (n=1)
- France, 2.4% (n=5)
- Germany, 14.7% (n=31)
- Israel, 4.7% (n=10)
- Italy, 4.3% (n=9)
- Russia, 1.4% (n=3)
- Spain, 9.5% (n=20)
- Sweden, 1.4% (n=3)
- United Kingdom, 19.4% (n=41)
- United States, 28.9% (n=61)

Breakdown of Enrolling Sites: Specialist Type

- Nephrologists (86%)
- Hematologists (14%)

Breakdown of Enrolling Sites: Adult versus Pediatric Centric

- Pediatric-centric sites (60%)
- Adult-centric sites (40%)

Table 1. Patient Demographics in Global aHUS Patient Registry (as of September 25, 2013)

	Ever Treated With Eculizumab (n=104)	Never Treated With Eculizumab (n=107)	Total (N=211)
Mean age at registry enrollment (SD), years	24.9 (21.00)	25.0 (17.22)	25.0 (19.17)
Age at registry enrollment, n (%)			
<2 years	17 (16.3)	2 (1.9)	19 (9.0)
≥2 to <5 years	8 (7.7)	7 (6.5)	15 (7.1)
≥5 to <12 years	15 (14.4)	18 (16.8)	33 (15.6)
≥12 to <18 years	5 (4.8)	16 (15.0)	21 (10.0)
≥18 years	55 (52.9)	55 (51.4)	110 (52.1)
N/A	4 (3.8)	9 (8.4)	13 (6.2)
Sex, n (%)			
Female	58 (55.8)	45 (42.1)	103 (48.8)
Male	46 (44.2)	54 (50.5)	100 (47.4)
N/A	0 (0.0)	8 (7.5)	8 (3.8)
Race, n (%)			
Asian	2 (1.9)	3 (2.8)	5 (2.4)
Black	5 (4.8)	1 (0.9)	6 (2.8)
Caucasian/African Caribbean	0 (0.0)	1 (0.9)	1 (0.5)
Caucasian	96 (92.3)	90 (84.1)	186 (88.2)
Latino	1 (1.0)	0 (0.0)	1 (0.5)
Mixed race (Black/Caucasian)	0 (0.0)	1 (0.9)	1 (0.5)
Mixed. Mother adopt	0 (0.0)	1 (0.9)	1 (0.5)
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N/A	0 (0.0)	9 (8.4)	9 (4.3)
Year of registry enrollment, n (%)			
2012	18 (17.3)	3 (2.8)	21 (10.0)
2013	86 (82.7)	104 (97.2)	190 (90.0)

aHUS, atypical hemolytic uremic syndrome; N/A, not available; SD, standard deviation.

Table 2. aHUS Diagnosis Characteristics at Registry Entry (as of September 25, 2013)

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Mean age at initial symptoms (SD), years	23.8 (21.36) n=93	17.1 (16.36) n=76	20.8 (19.51) n=169
Mean age at diagnosis (SD), years	23.9 (21.44) n=94	17.1 (16.79) n=75	20.9 (19.75) n=169
Stated family history of aHUS, n (%)			
Yes	16 (15.4)	20 (18.7)	36 (17.1)
No	88 (84.6)	87 (81.3)	175 (82.9)
Any identified complement genetic mutation or auto-antibody, n (%)			
N/A	16 (15.4)	38 (35.5)	54 (25.6)
No	41 (39.4)	24 (22.4)	65 (30.8)
Yes	47 (45.2)	45 (42.1)	92 (43.6)

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Mean baseline eGFR (SD), mL/min/1.73 m^2	42.8 (48.11) n=39	76.0 (63.81) n=29	57.0 (57.34) n=68

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Median dose at initiation of eculizumab (range), mg	900.0 (300–1200)
Mean time on eculizumab (SD), years	1.2 (0.89) n=100
Any discontinuation of eculizumab, n (%)	
No	86 (83.5)
Yes	17 (16.5)
Restarted eculizumab (among those who discontinued), n (%)	
No	14 (82.4)
Yes	3 (17.6)

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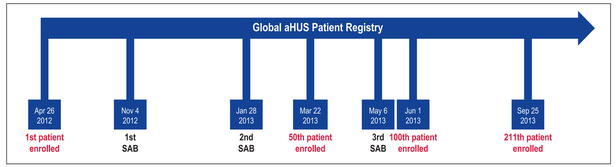
Milestones Achieved for the Global aHUS Patient Registry

- **Figure 1** shows the milestones that have been reached to date since enrollment of the first patient on April 26, 2012.

Figure 1. aHUS Patient Registry: A Time Line of Milestones Reached to Date



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CONCLUSIONS

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- New clinical sites are encouraged to participate.
- As of September 25, 2013, a total of 211 patients have enrolled in the global aHUS patient registry.

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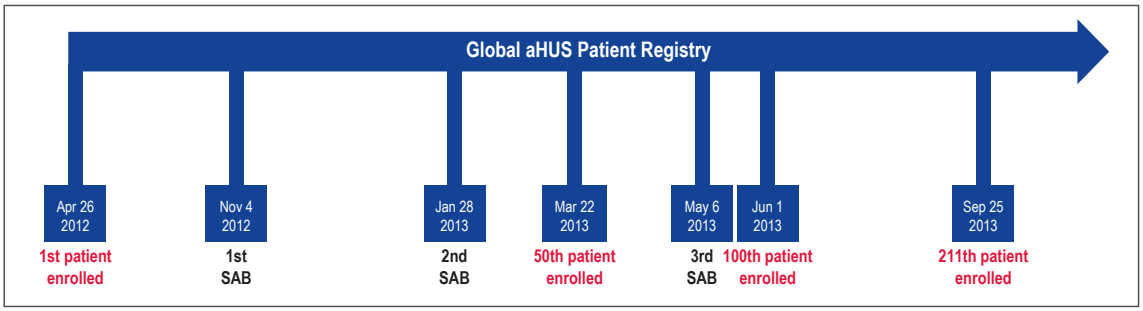

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